NARCOLEPSY NETWORK
10921 Reed Hartman Highway – Suite 119
Cincinnati, Ohio 45242

Tel: (513) 891-3522 **Fax: (513) 891-3836** **E-mail: narnet@aol.com**

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Peripheral and Central Nervous System Drugs Advisory Committee
c/o Sandra L. Titus
Center for Drug Evaluation and Research (HFD–21)
US Food and Drug Administration
5600 Fishers Lane
Rockville, MD 20857
e-mail: tituss@cder.fda.gov

Subject: New Drug Application 21-196
Xyrem (Sodium Oxybate)

Dear Committee Members and Ms. Titus:

Thank you for this opportunity to address your study of safety, efficacy, and risk management issues regarding Xyrem (a.k.a. GHB). I will briefly inform you of both my long personal use of GHB, and my very serious concerns as Executive Director of Narcolepsy Network. My hope and purpose are that you will allow the estimated 25,000 Americans who suffer from the disabling cataplexy symptom of narcolepsy the same positive and even life-saving benefits I have received from this product.

First, my personal experience with GHB as a narcolepsy patient. I am 57 years old, married, with two adult children, and am an attorney in private practice (family and criminal law). **I may be the first American to have used GHB for narcolepsy, and the longest continuing user of this drug, now approaching 19 years.** My narcolepsy/cataplexy symptoms first appeared in my mid-30's, and by age 39 included severe and recurring cataplexy, together with excessive daytime sleepiness and sudden recurring sleep attacks. My cataplexy was causing numerous daily episodes of complete body collapse, such that I could no longer even walk from my home or office without serious risk of harm to myself

and others. Feeling any emotion (humor, anger, and even mere surprise or enthusiasm) would cause me to suddenly collapse like a puppet without strings. I would usually fall backwards, with my head whipping down last on concrete, metal, table corners, stairs – whatever was there. I have often been "rescued" by emergency crews, police, lifeguards, strangers and friends. Some falls resulted in hospital visits for injuries, fortunately none permanent. But there are others whose falls have been fatal. Moreover, I would fall suddenly into REM sleep, even in mid-sentence. Disability was staring me in the face.

Then, in August 1982 my treating doctor sent me to Sunnybrook Medical Center in Toronto, Canada to begin prescriptive use of GHB under the research studies being conducted by Dr. Mortimer Mamelak. After three weeks, I returned home and continued using GHB, as monitored by my local physician under an approved FDA individual investigational new drug application. My significant cataplexy and sudden sleep attacks disappeared almost overnight. I was immediately able to return to my full-time law practice. Since then, I have continued using GHB under the FDA clinical investigative procedures conducted first by my local physician, and in recent years by Orphan Medical. **During these 19 years, I have never changed the dose, have never experienced tolerance, and have noted no side effects. Simply stated, the drug is as safe and effective now as it was at the start.** (Frankly, it is difficult to imagine a pharmaceutical product offering such quick, complete, safe and enduring benefits.)

Secondly, my privileged service as Executive Director of Narcolepsy Network in recent years is motivated by the effective medical treatment I received, and a desire that others with narcolepsy might be as fortunate. Narcolepsy Network is a national nonprofit organization whose mission is to educate the public, healthcare professionals, and government representatives regarding this disabling neurological disease, and to facilitate more prompt, informed and effective treatment for persons with narcolepsy. We work closely with the National Center for Sleep Disorder Research at the National Institute of Health,

the American Academy of Sleep Medicine, the National Sleep Foundation, sleep disorder centers as well as Orphan Medical and other pharmaceutical companies developing orphan products for narcolepsy. We have sought to inform federal and state government officials, whenever appropriate, of the dramatic medical benefits provided by GHB to patients participating in the clinical trials. A fortunate result has been the present bifurcated scheduling of GHB on the federal level and in many states. I have often stated to congressional committees and legislative representatives over recent years that the greatest tragedy in the development of treatments for narcolepsy has been the unavailability of GHB, in prescriptive form, to other patients like myself with narcolepsy and cataplexy. Now I respectfully ask this committee to assist in eliminating such an unnecessary situation.

Finally, we are very mindful of and cannot ignore the injuries, deaths, and other "victimizations" which many young Americans have suffered from unlawful and/or uncontrolled consumption of GHB or its related chemical compounds. Narcolepsy Network and myself have cooperated extensively with law enforcement agencies, medical professionals, and community drug agencies. Our continuing purpose is to minimize unlawful use of GHB, and to design safeguards to reduce access and availability. These concerns deserve your and our utmost attention.

However, equally deserving of your highest consideration is the promise and potential of medically controlled GHB to allow persons with narcolepsy with severely disrupted lives and frequent disability to again rejoin their jobs, communities and families.

Thank you for your professional consideration.

Respectfully,

Robert L. Cloud
Executive Director
Narcolepsy Network, Inc.